

## California stem cell and gene therapy agency investing in clinical trial for knee osteoarthritis

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The governing Board of the California Institute for Regenerative Medicine (CIRM) voted to invest \$15,637,174 in two projects in the agency's Clinical program at its February meeting. These awards bring the number of CIRM-funded clinical trials to 88.

Included in the new awards is an \$11.6 million grant to support Genascence in a Phase 1b placebo-controlled clinical trial to evaluate the safety and tolerability of a gene therapy for knee osteoarthritis, a degenerative joint disease that is a leading cause of disability.

Osteoarthritis—which affects more than 30 million Americans—specifically causes the breakdown of cartilage tissue in the joints. It does not just strike the elderly: 14 percent of people over 25 have the degenerative joint disease. That rises to 33 percent for those over 65 with the end-stage disease, resulting in a million joint replacement surgeries each year. Current treatments for the joint disease cause only temporary relief. Nothing can slow or stop osteoarthritis progression.

The gene therapy being tested in the Genascence clinical trial is called GNSC-001, and aims to block a molecule called IL-1, which plays a key role in the development of osteoarthritis and regulating inflammation.

In this trial, 40 osteoarthritis patients will have the product injected to the knee joint, while 10 will receive a placebo injection. If successful, the treatment will reduce inflammation and pain, and may slow or reverse disease progression (cartilage degeneration).

"Genascence's novel gene therapy approach could usher in a step-change in how we treat osteoarthritis and other musculoskeletal diseases, which are among the leading causes of disability in California and around the world," said Dr. Maria T. Millan, President and CEO of CIRM.

Dr. Thomas Chalberg, Founder and CEO of Genascence, added, "We are honored to have CIRM award this funding to help advance GNSC-001, our lead clinical program in osteoarthritis where we hope to demonstrate transformative results for patients. We are unwavering in our commitment to developing novel therapies for widespread, debilitating diseases like osteoarthritis."

The list of successful applicants in CIRM's Clinical program this month are:

| Application | Program Title   | Institution - Principal Investigator       | Amount Awarded |
|-------------|---|--|----------------|
| CLIN2-14265 | A Phase 1b, Randomized, Blinded, Placebo-Controlled Dose-Ranging Study of GNSC-001 Evaluating Safety, Pharmacodynamics, and Biomarkers in Knee OA | Genascence Corporation - Thomas W Chalberg | \$11,637,194   |
| CLIN1-14299 | Ex vivo Engineering of Autologous Hematopoietic Stem Cells for the Treatment of Hypophosphatasia  | Rampart Bioscience - Jeffrey Bartlett      | \$3,999,980    |

The purpose of CIRM CLIN2 awards is to support completion of a clinical trial for a regenerative medicine-based therapy (stem cell-based or gene therapy) that addresses an unmet medical need.

CIRM's CLIN1 awards support late-stage preclinical studies necessary to file and attain an active Investigational New Drug (IND) application or Investigational Device Exemption (IDE) with the Food and Drug Administration (FDA) to prepare for a clinical trial in patients.

CIRM has invested more than \$30 million in several approaches aiming to stop or reverse osteoarthritis. Visit our website to learn more.

**[About the California Institute for Regenerative Medicine \(CIRM\)](#)**

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is one of the world's largest institutions dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to [www.cirm.ca.gov](http://www.cirm.ca.gov)

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